

# A Novel, Regulated Gene Therapy (NGN-401) Study for Females With Rett Syndrome

NCT05898620

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Status	RECRUITING
Phase	Phase 3
Sponsor	Neurogene Inc.
Enrollment	33 participants

## Key Eligibility Criteria

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### Inclusion (6)

- Females who are between the ages of e4 and d10 years for Arms 1 and 2 (Arms closed). Females who are e11 years of age or older for Arm 3 (Arm closed). Females who are e3 for Arm 4, the pivotal cohort.
- Diagnosis of typical Rett syndrome with a documented disease-causing mutation in the methyl-CpG-binding protein 2 (MECP2) gene
- Current anti-epileptic drug regimen has been stable for at least 12 weeks
- Participant must be in the post-regression stage
- Participant and caregiver should reside within a 2-hour drive of the study center for at least 3 months following treatment
- ... and 1 more (see full listing online)

### Exclusion (3)

- Normal or near normal hand function
- Has a current clinically significant condition other than Rett syndrome
- Presence of a concomitant medical condition that precludes intracerebroventricular administration, or use of anesthetics or immune suppression needed for study related procedures

## Locations (16 total)

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University of Alabama at Birmingham, Birmingham, Alabama, United States  
UCSF Benioff Children's Hospital Oakland, Oakland, California, United States  
Children's Hospital Colorado, Aurora, Colorado, United States  
... and 13 more locations